A Phase I/II, Non Randomized, Multicenter, Open-Label Study of G1XCGD (Lentiviral Vector Transduced CD34+ Cells) in Patients With X-Linked Chronic Granulomatous Disease

Grant Award Details

A Phase I/II, Non Randomized, Multicenter, Open-Label Study of G1XCGD (Lentiviral Vector Transduced CD34+ Cells) in Patients With X-Linked Chronic Granulomatous Disease

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-08231

Project Objective: To conduct a Phase 1/2 non-randomized, multicenter, open-label study of G1XCGD (lentiviral vector transduced autologous CD34+ cells in patients with X-linked chronic granulomatous disease. A total of 10 subjects are planned to be enrolled . There are 3 clinical sites involved in this study - UCLA, Boston Children and NIH clinical center.

Investigator:

Name:	Donald Kohn
Institution:	University of California, Los Angeles
Type:	PI

Disease Focus: Immune Disease, Pediatrics, X-linked Chronic Granulomatous Disease, Blood Disorders

Human Stem Cell Use: Adult Stem Cell

Cell Line Generation: Adult Stem Cell

Award Value: $7,083,364

Status: Active

Progress Reports

Reporting Period: Operational Milestone #1

View Report

Grant Application Details

Application Title: A Phase I/II, Non Randomized, Multicenter, Open-Label Study of G1XCGD (Lentiviral Vector Transduced CD34+ Cells) in Patients With X-Linked Chronic Granulomatous Disease

Public Abstract:

Therapeutic Candidate or Device

The therapeutic product candidate is autologous CD34+ hematopoietic stem/progenitor cells (HSPC) transduced with the G1XCGD lentiviral vector.

Indication

The target indication is for the transplantation of patients with severe X-linked Chronic Granulomatous Disease (XCGD) lacking matched donors.

Therapeutic Mechanism

Transplantation and engraftment of gene-corrected autologous HSPC after reduced intensity conditioning for XCGD may lead to sustained production of white blood cells that have the capacity to kill infectious microorganisms and eliminate or prevent severe recurrent infections. Autologous transplants may be as effective and safer than transplants from an unrelated donor to alleviate disease manifestations.

Unmet Medical Need

Hematopoietic stem cell transplantation (HSCT) from non-fully matched donors may have immune complications and requires potent immune suppression. Effective autologous HSCT with G1XCGD lentiviral vector-mediated gene correction could have similar benefits but be safer with less complications.

Project Objective

The objective is to complete a Phase I/II trial.

Major Proposed Activities

- Perform two year follow up to assess trial end-points to assess safety and efficacy.
- Transplant 10 subjects with severe XCGD lacking matched donors with the autologous stem cell product after reduced intensity conditioning.
- Perform GMP manufacture of 10 patient-specific lots of G1XCGD transduced autologous CD34+ HSPC meeting release criteria.

Statement of Benefit to California:

Patients with primary immune deficiency diseases, such as XCGD, have severe and recurrent infections, necessitating extensive medical therapy and leading to significant morbidity and early mortality. Transplantation of their own hematopoietic stem cells corrected with the normal gene may provide cures for XCGD as well as other inherited blood cell disorders (SCID or "bubble baby disease", sickle cell disease, Lorenzo's Oil disease), and potentially provide novel curative approaches to HIV/AIDS.

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